Catalyzing Translational Innovation

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NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES
NATIONAL INSTITUTES OF HEALTH

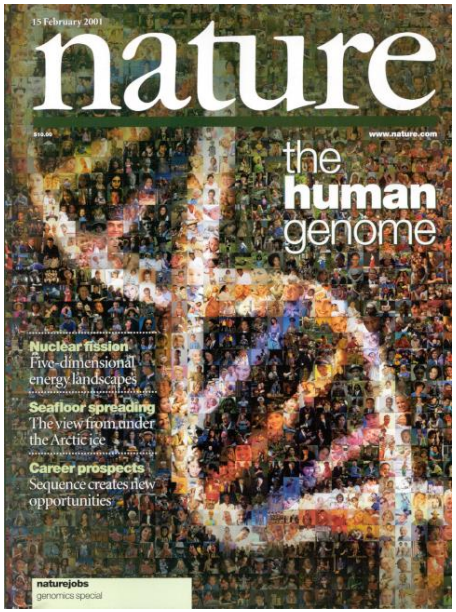
HEALTH RESEARCH ALLIANCE MEMBERS' MEETING
SEPTEMBER 28, 2016

NCATS

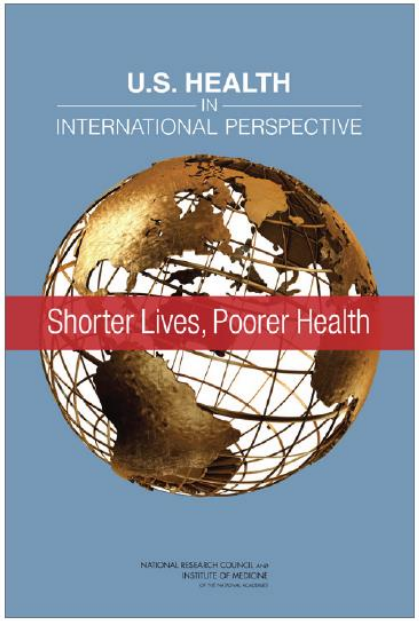
NIH National Center for Advancing Translational Sciences

The Best of Times, the Worst of Times

Fundamental science unprecedentedly advanced, but:



- Poor transition of basic or clinical observations into interventions that tangibly improve human health
- Drug/device/diagnostic development expensive and failure-prone
- Clinical trials system inefficient
- Poor adoption of demonstrably useful interventions

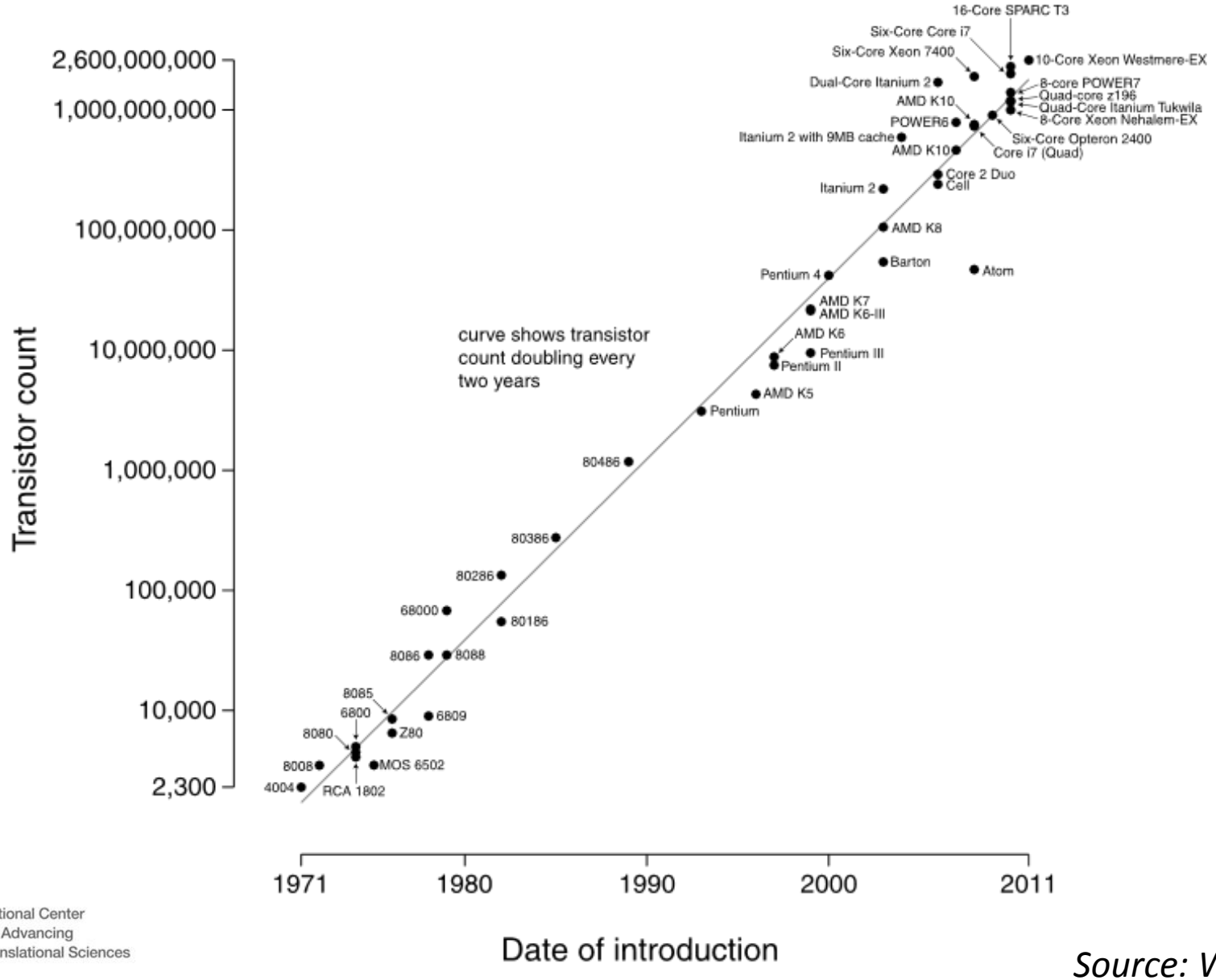


People unhealthier and funders of biomedical research enterprise (public and private) impatient

Human Conditions with Known Molecular Basis

Source: Online *Mendelian Inheritance in Man*, Morbid Anatomy of the Human Genome

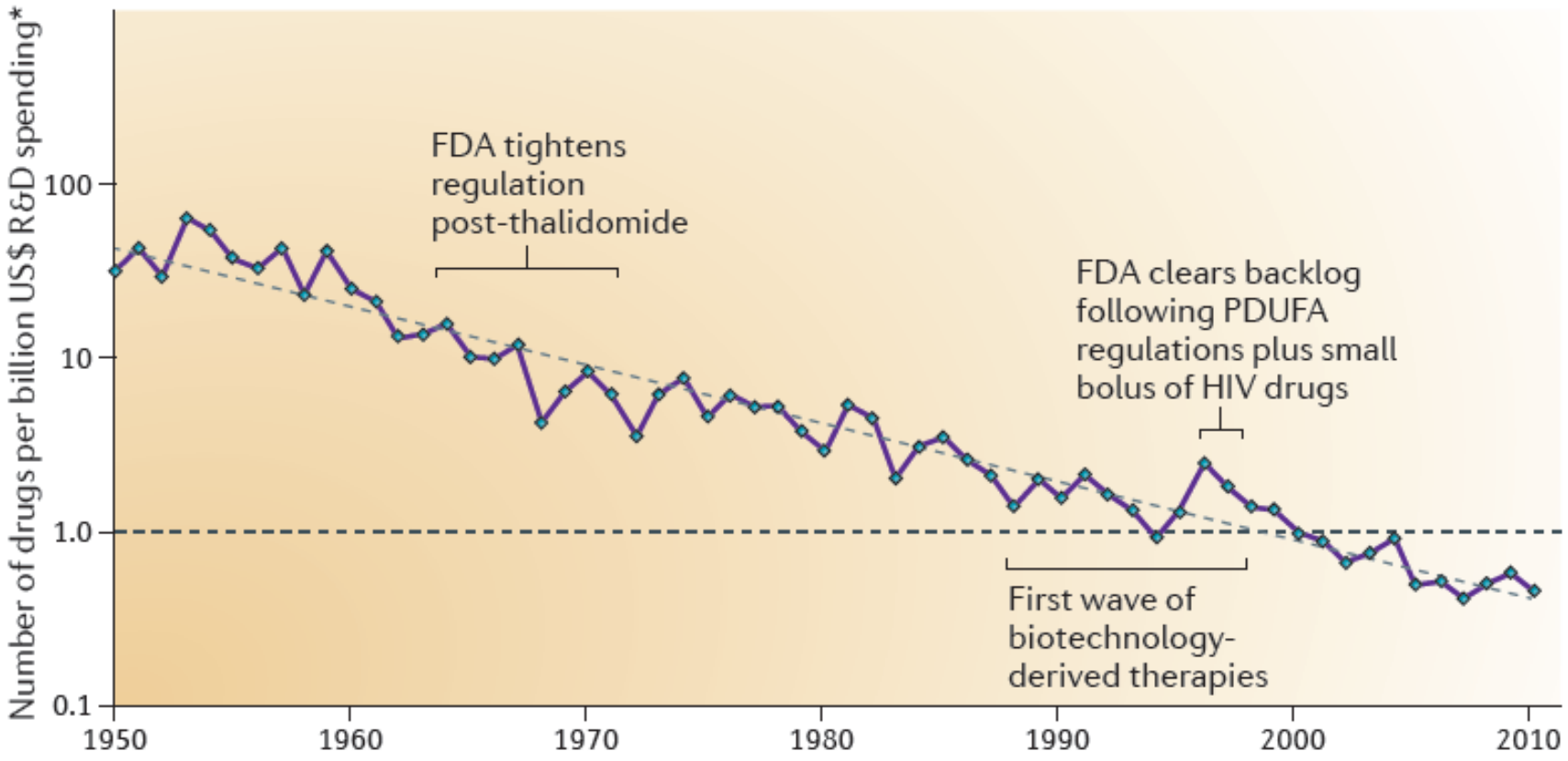
Moore's Law



National Center for Advancing Translational Sciences

Source: Wikipedia

Eroom's Law



The number of new drugs approved by the FDA per billion US dollars (inflation-adjusted) spent on research and development (R&D) has halved roughly every 9 years since 1950.

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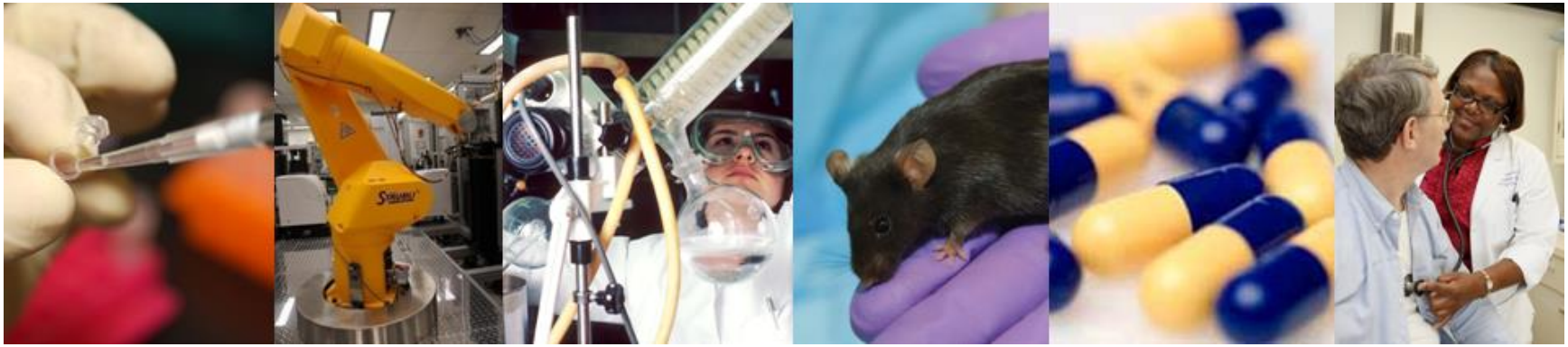
Scannell et al., *Nature Reviews Drug Discovery* 11:191, 2012

NCATS Mission



To catalyze the generation of innovative methods and technologies that will enhance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions.

What is Translation?

Translation is the process of turning observations in the laboratory, clinic, and community into interventions that improve the health of individuals and the public - from diagnostics and therapeutics to medical procedures and behavioral changes.

What is Translational Science?

Translational Science is the field of investigation focused on understanding the scientific and operational principles underlying each step of the translational process.

NCATS studies translation as a scientific and organizational problem.

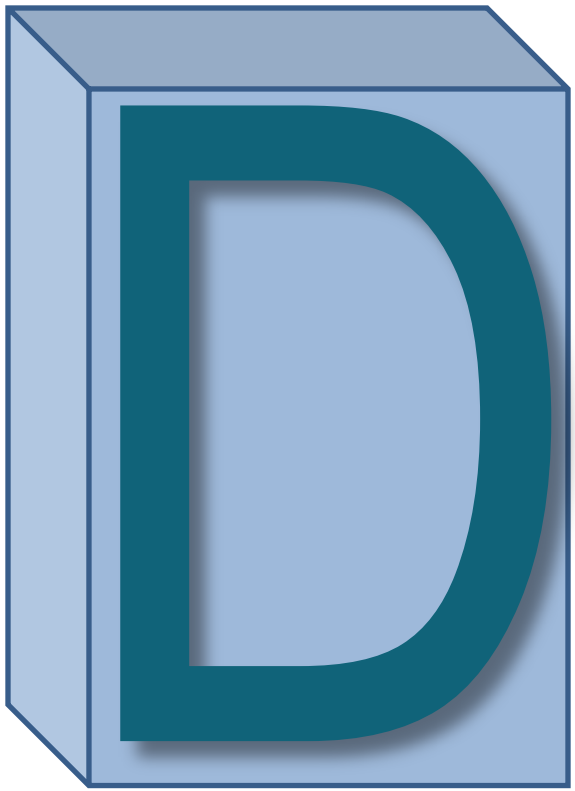
Some of the scientific translational problems on NCATS' to-do list

- Predictive toxicology
- Predictive efficacy
- Derisking undruggable targets/untreatable diseases
- Data interoperability
- Biomarker qualification process
- Clinical trial networks
- Patient recruitment
- Electronic Health Records for research
- Harmonized IRBs
- Clinical diagnostic criteria
- Clinical outcome criteria (e.g., PROs)
- Adaptive clinical trial designs
- Shortening time of intervention adoption
- Methods to better measure impact on health (or lack of)

Some of the organizational translational problems on NCATS' to-do list…

- Data transparency/release
- IP management
- Integration of project management
- Incentives/credit for team science
- Incentives/credit for health improvements
- Education/Training (scientific and cultural)
- Collaborative structures
  - Public-private partnership models

NCATS "3D's"



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NCATS Scientific Initiatives

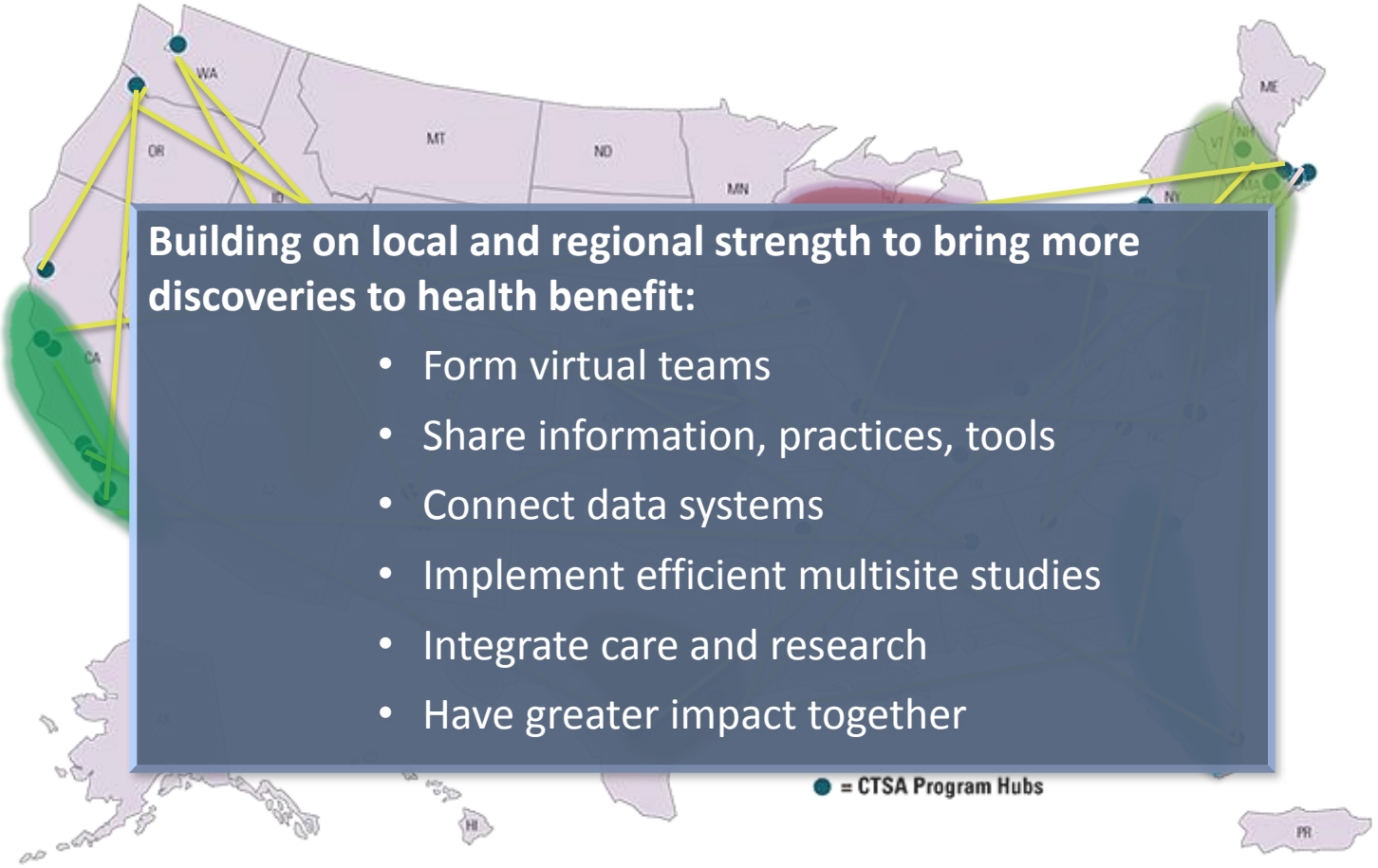
- **Clinical Translational Science**
  - » Clinical and Translational Science Awards
  - » Rare Disease Clinical Research Network
  - » New Therapeutic Uses program
- **Preclinical Translational Science**
  - » NCATS Chemical Genomics Center
  - » Therapeutics for Rare and Neglected Diseases program
  - » Bridging Interventional Development Gaps program
- **Re-engineering Translational Sciences**
  - » Toxicology in the 21st Century
  - » Microphysiological Systems (Tissue Chip) program
  - » Office of Rare Diseases Research

Division of Clinical Innovation

Clinical and Translational Science Awards (CTSA) Program

- A national consortium of medical research institutions
- Improves the way clinical and translational research is conducted nationwide
- Accelerates the research translation process
- Provides innovative training for clinical and translational researchers

Collaborative Consortium for Translational Research



Building on local and regional strength to bring more discoveries to health benefit:

- Form virtual teams
- Share information, practices, tools
- Connect data systems
- Implement efficient multisite studies
- Integrate care and research
- Have greater impact together

NCATS Trial Innovation Network

The Network will optimize clinical trials enterprise to accelerate translation

- Data-driven "learning clinical studies system" so trials can recruit more quickly, retain participants, finish on-time and on-budget, and produce impactful, high-quality data
- Leverage the talent, expertise & resources of the CTSA Program to transform clinical trials

Key components of the Network were awarded in July 2016

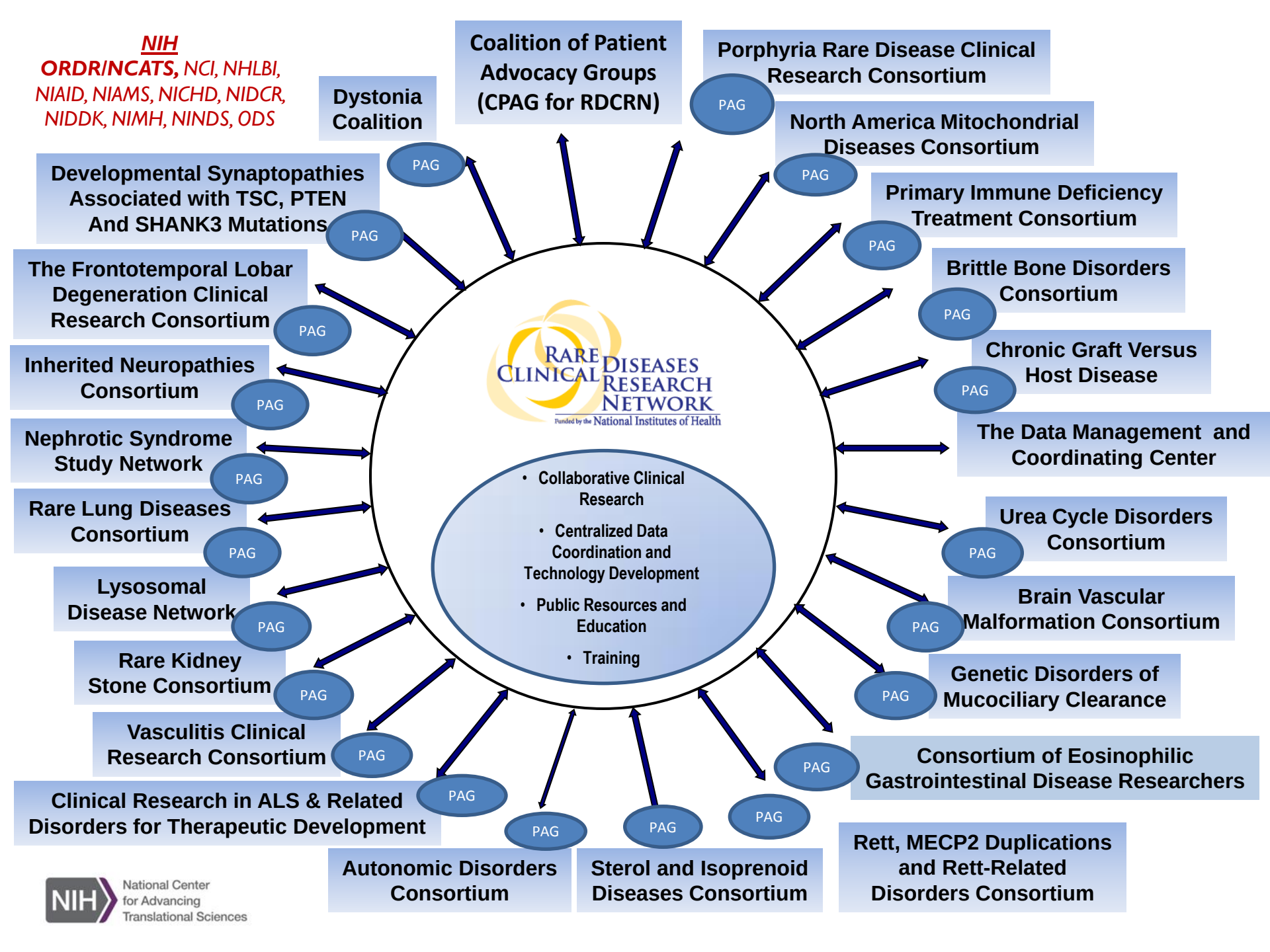
- **Recruitment Innovation Center (RIC):** develop and implement strategies to engage patients and communities in clinical trials
- **Trial Innovation Centers (TICs):** IRB, contracting, GCP training to improve efficiency and effectiveness of clinical studies

Some examples of CTSA Partnerships with Foundations

CTSA	Engagement	Foundation Collaborator(s)
Rockefeller University	Pilot projects in digestive disease research, bio-medicine, and nutrition	Dracopolous Foundation; Helmsley Charitable Trust; Jumming Le Foundation; Niarchos Foundation; Roberston Foundation; Sackler Foundation; Vilcek Foundation
University of Washington	Program to organize and conduct free, monthly cardiovascular screenings at Seattle-area high schools	Nick of Time Foundation
University of Wisconsin–Madison	Pilot project to deliver family-centered diabetes self-management resources Young investigator research training support	Juvenile Diabetes Research Foundation Cystic Fibrosis Foundation; Michael J. Fox Foundation
Washington University	Pilot projects, training awards	Barnes-Jewish Hospital Foundation

Office of Rare Diseases Research

- **Rare Diseases Clinical Research Network (RDCRN)**
  - 22 consortia at 250 institutions worldwide
  - Studying >200 diseases with 83 active protocols, and
  - More than 85 patient advocacy groups participating
- **Genetic and Rare Disease Information Center (GARD)**
- **Scientific Conferences Program**
  - Identify scientific opportunities and establish research agendas
  - Patients + NCATS + NIH ICs + FDA + Biopharma
- **Global Rare Disease Registry (GRDR)**
  - 15 GRDR patient registries + 19 existing registries
  - Ability to conduct cross-disease analysis and recruitment


NIH
ORDR/NCATS, NCI, NHLBI, NIAID, NIAMS, NICHD, NIDCR, NIDDK, NIMH, NINDS, ODS
Coalition of Patient Advocacy Groups (CPAG for RDCRN)
Porphyria Rare Disease Clinical Research Consortium
Dystonia Coalition
North America Mitochondrial Diseases Consortium
Developmental Synaptopathies Associated with TSC, PTEN And SHANK3 Mutations
Primary Immune Deficiency Treatment Consortium
The Frontotemporal Lobar Degeneration Clinical Research Consortium
Brittle Bone Disorders Consortium
Inherited Neuropathies Consortium
Chronic Graft Versus Host Disease
Nephrotic Syndrome Study Network
The Data Management and Coordinating Center
Rare Lung Diseases Consortium
Urea Cycle Disorders Consortium
Lysosomal Disease Network
Brain Vascular Malformation Consortium
Rare Kidney Stone Consortium
Genetic Disorders of Mucociliary Clearance
Vasculitis Clinical Research Consortium
Consortium of Eosinophilic Gastrointestinal Disease Researchers
Clinical Research in ALS & Related Disorders for Therapeutic Development
Autonomic Disorders Consortium
Sterol and Isoprenoid Diseases Consortium
Rett, MECP2 Duplications and Rett-Related Disorders Consortium
PAG
RARE CLINICAL DISEASES RESEARCH NETWORK
Funded by the National Institutes of Health
• Collaborative Clinical Research
• Centralized Data Coordination and Technology Development
• Public Resources and Education
• Training
NIH National Center for Advancing Translational Sciences

RDCRN Partnerships with Foundations

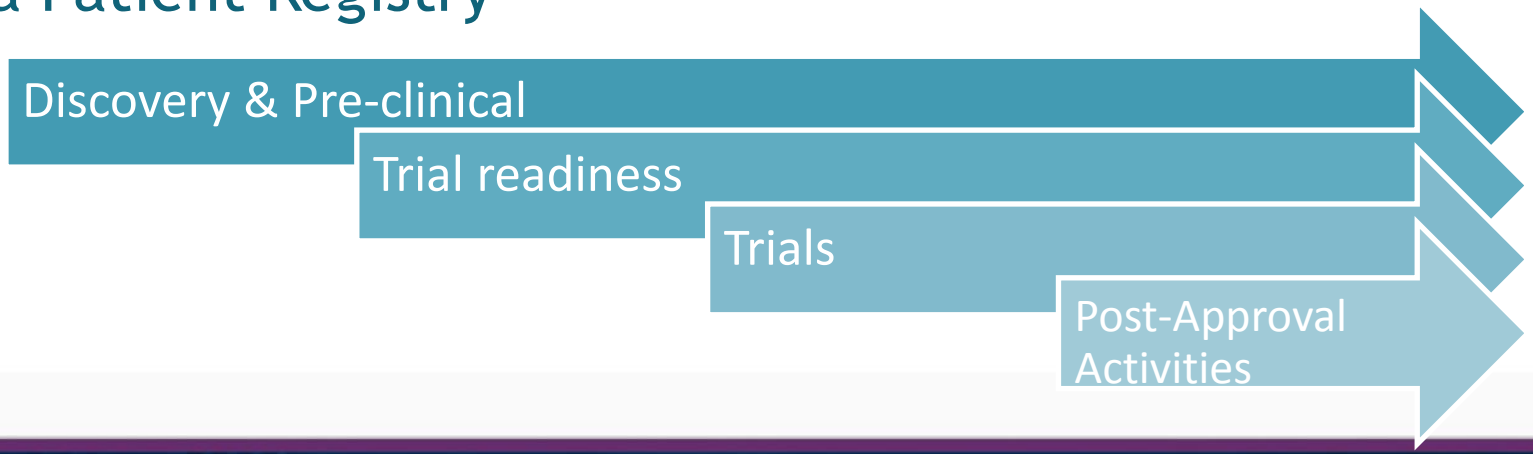
Consortium	Foundation Collaborator(s)
Advancing Research and Treatment for Frontotemporal Lobar Degeneration	Foundation for PSP CBD and Related Brain Disease
Autonomic Disorders	National Dysautonomia Research Foundation
Brain Vascular Malformation	Sturge-Weber Foundation
Brittle Bone Disorders	Osteogenesis Imperfecta Foundation
Clinical Research in Amyotrophic Lateral Sclerosis and Related Disorders	Spastic Paraplegia Foundation
Developmental Synaptopathies Consortium	PTEN Hamartoma Tumor Syndrome Foundation; Phelan-McDermid Syndrome Foundation
Dystonia Coalition	Dystonia Medical Research Foundation;
Genetic Disorders of Mucociliary Clearance Consortium	Bronchiectasis Research Registry/COPD Foundation; Cystic Fibrosis Foundation; Heterotaxy Foundation; PCD Foundation
Inherited Neuropathies Consortium	Charcot Marie Tooth Association; Hereditary Neuropathy Foundation
Lysosomal Disease Network	Adrenoleukodystrophy Foundation; Ara Parseghian Medical Research Foundation; Ben's Dream Sanfilippo Research Foundation; Cystinosis Foundation; Hide and Seek Foundation for Lysosomal Disease Research; Hunter's Hope Foundation; Mucolipidosis IV Foundation; Nathan's Battle Foundation; National Fabry Disease Foundation; National Gaucher Foundation; National Niemann-Pick Disease Foundation; Ryan Foundation; United Leukodystrophy Foundation

RDCRN Partnerships (cont'd)

Consortium	Foundation Collaborator(s)
Nephrotic Syndrome Study Network	Halpin Foundation
North American Mitochondrial Disease Consortium	United Mitochondrial Disease Foundation
Porphyrias Consortium	American Porphyria Foundation
Primary Immune Deficiency Treatment Consortium	Immune Deficiency Foundation; Jeffrey Modell Foundation; Wiscott-Aldrich Foundation
Rare Kidney Stone Consortium	International Cystinuria Foundation; Oxalosis and Hyperoxaluria Foundation
Rare Lung Diseases Consortium	Alpha-1 Foundation; Children's Interstitial and Diffuse Lung Disease Foundation; Lymphangioleiomyomatosis Foundation; Pulmonary Alveolar Proteinosis Foundation; Sjogren's Syndrome Foundation
Rett Syndrome, MECP2 Duplications, and Rett-related Disorders Consortium	International Foundation for CDKL5 Research; International FOXG1 Foundation
Sterol & Isoprenoid Research Consortium	Ara Parseghian Medical Research Foundation; Foundation for Ichthyosis and Related Skin Types; Global Foundation for Peroxisomal Disorders; Sitosterolemia Foundation; Smith Lemli Opitz/RSH Foundation; United Leukodystrophy Foundation
Urea Cycle Disorders Consortium	National Urea Cycle Disorders Foundation
Vasculitis Clinical Research Consortium	Lauren Currie Twilight Foundation; Vasculitis Foundation

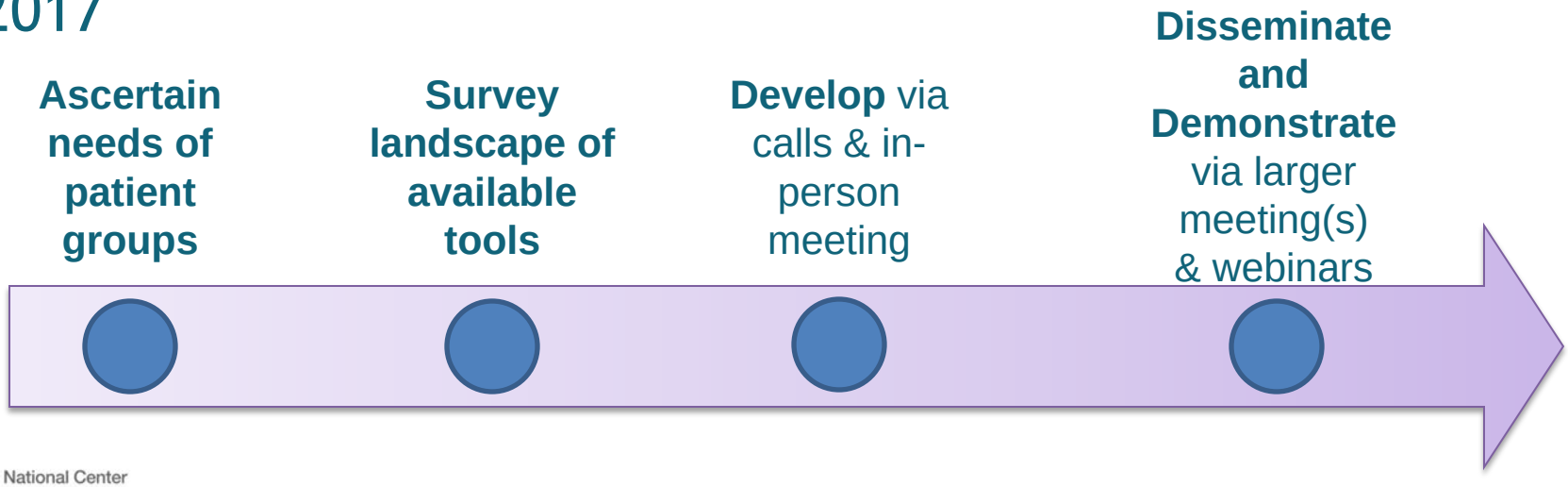
Rare Disease Patient Toolkit Project

- Provide centralized web portal to online tools and resources that patient groups can readily access to accelerate their work
- Focus on tools/resources across the drug development process
- "How-to" perspective, e.g. "How To Establish and Utilize a Patient Registry"



RD Patient Toolkit Being Developed though Community Engagement

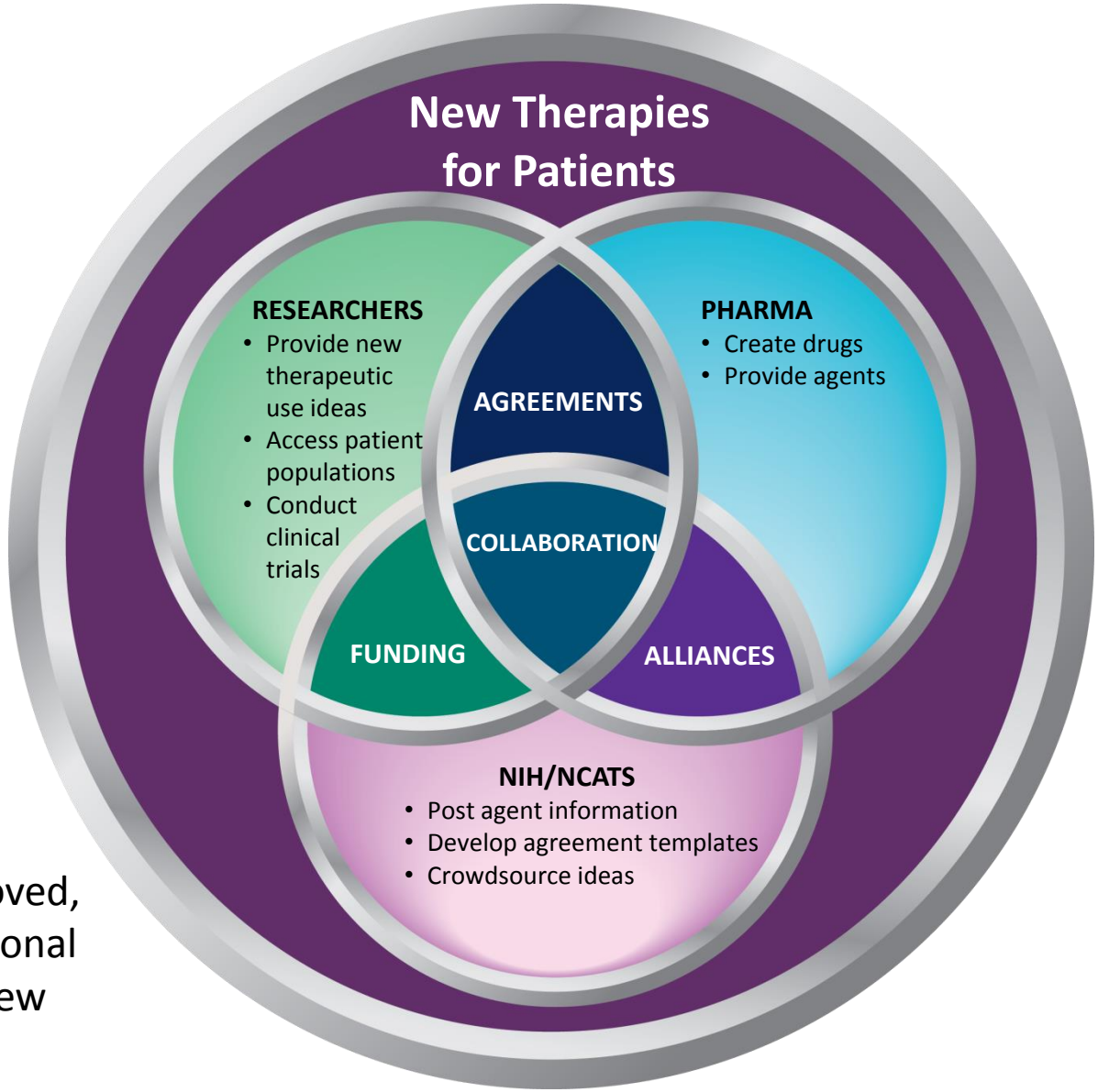
- Working groups that represent diverse rare disease stakeholder community
- Conducting landscape analysis of current tools and resources for each phase of drug development process
- Gaps identified will be filled with new tool development
- Prototype demonstration/dissemination meeting Spring 2017



New Therapeutic Uses Program

80% of drugs that enter clinic are never approved

For every 1 drug approved, 4 late-stage investigational drugs available for new indication finding



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New Therapeutic Uses Program

- 9 projects in 8 diseases funded in Round 1

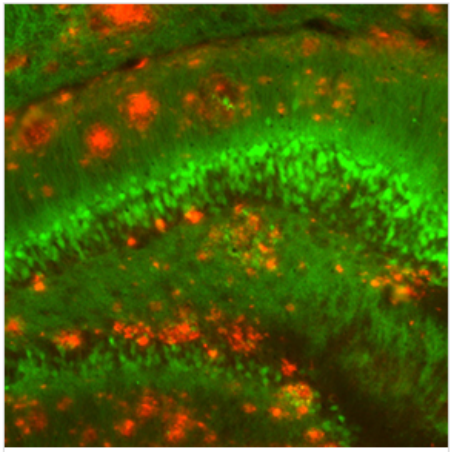
Disease	Academic Partner	Pharma Partner
Alzheimer's Disease	Yale	AstraZeneca
Alcoholism	U Rhode Island/NIAAA	Pfizer
Calcific Aortic Stenosis	Mayo Clinic	Sanofi
Duchenne Muscular Dystrophy	Kennedy Krieger/UWash	Sanofi
Lymphangioleiomyomatosis	Baylor	AstraZeneca
Peripheral Artery Disease	U Virginia	AstraZeneca
Schizophrenia	Yale	Pfizer
Schizophrenia	Indiana U	Lilly
Smoking Cessation	VCU/Pittsburgh	Janssen

- Translational Innovation Success Measures
  - Does use of template agreements speed negotiation time?
  - Does crowdsourcing of indications generate new ideas?
  - Do studies result in new indications/approvals?

News Releases & Announcements
Media Coverage
Grantee & Partner News
Events
Feature Stories
Scientific Publications
Media Resources
e-Newsletter and Listserv

NCATS Support Leads to Clinical Trial to Test Repurposed Cancer Treatment as Alzheimer's Therapy

As Baby Boomers get older, the number of people with age-related conditions such as cancer and Alzheimer's disease continues to grow. Alzheimer's disease is the most common form of dementia, a group of disorders that cause progressive loss of memory and other mental processes. About 5 million Americans have Alzheimer's disease, and current drug therapies can only ease symptoms of the disease without stopping its progression. New treatments — so-called disease-modifying therapies — are needed to halt Alzheimer's by targeting its underlying mechanisms.



In a mouse model of Alzheimer's disease, amyloid beta clusters (red) build up among neurons (green) in a memory-related area of the brain. (Strittmatter Laboratory, Yale University Photo/Adam Kaufman)

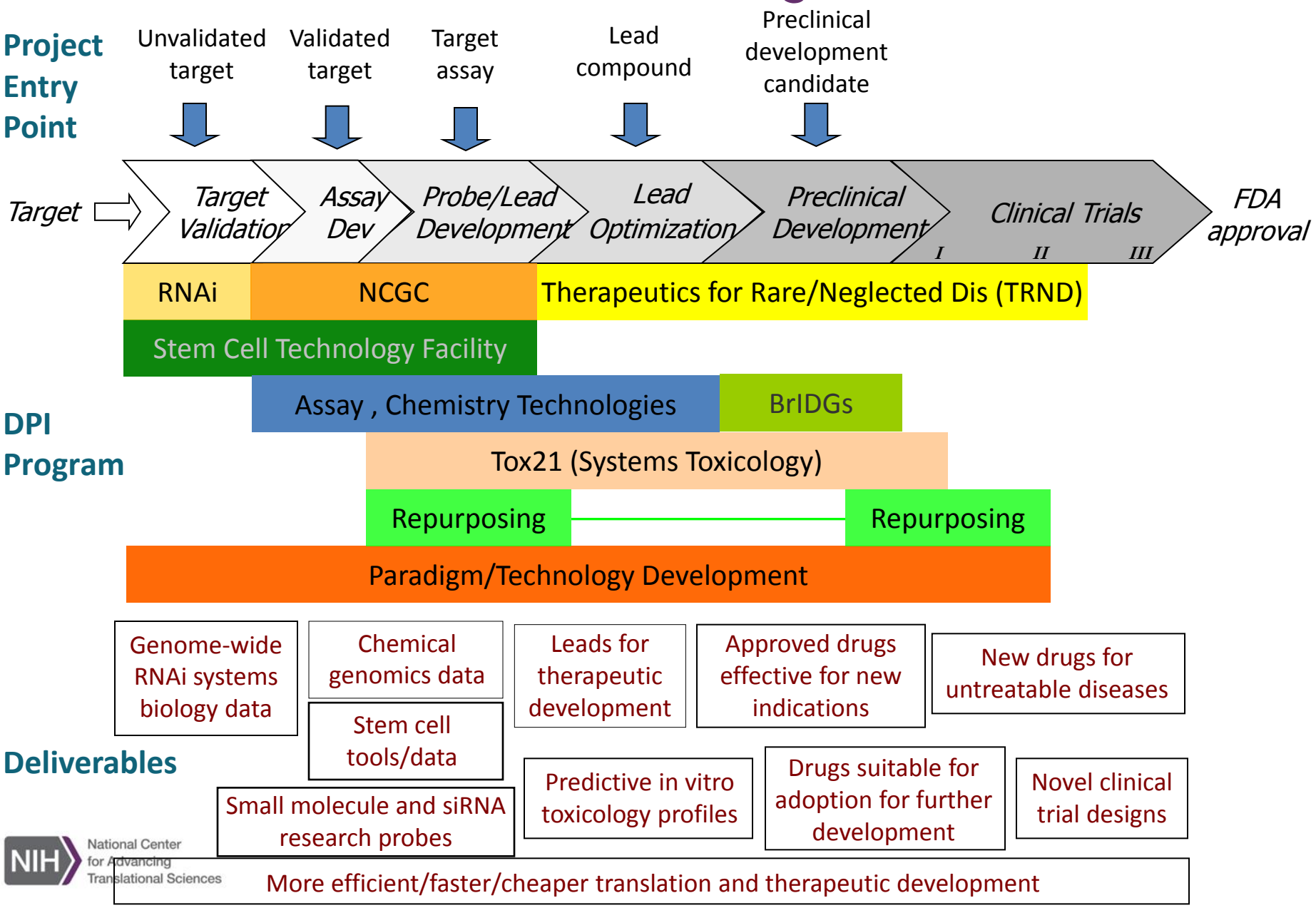
Blocking that path to therapeutic success is the costly, complex process of drug development. The average length of time from discovery of a therapeutic target to approval of a new drug is about 14 years. The failure rate during this process exceeds 95 percent.

NCATS is addressing these translational bottlenecks through programs such as the Discovering New Therapeutic Uses for Existing Molecules (New Therapeutic Uses) program. Launched in 2012, this initiative matches academic researchers with pharmaceutical industry assets that have undergone significant research and development to accelerate the process of finding new therapies.

Now, NCATS is celebrating one of the first promising results from the New Therapeutic Uses program: Center-supported scientists at Yale University School of Medicine have found that an experimental compound originally developed as a cancer therapy potentially could be used to treat Alzheimer's disease. The compound successfully reversed brain problems in mouse models of the condition, and now the researchers are testing it in humans. The results of the animal study were published for early view on March 21, 2015, in the *Annals of Neurology*. Read the NIH news release.

NCATS Division of Preclinical Innovation

A Collaborative Engine



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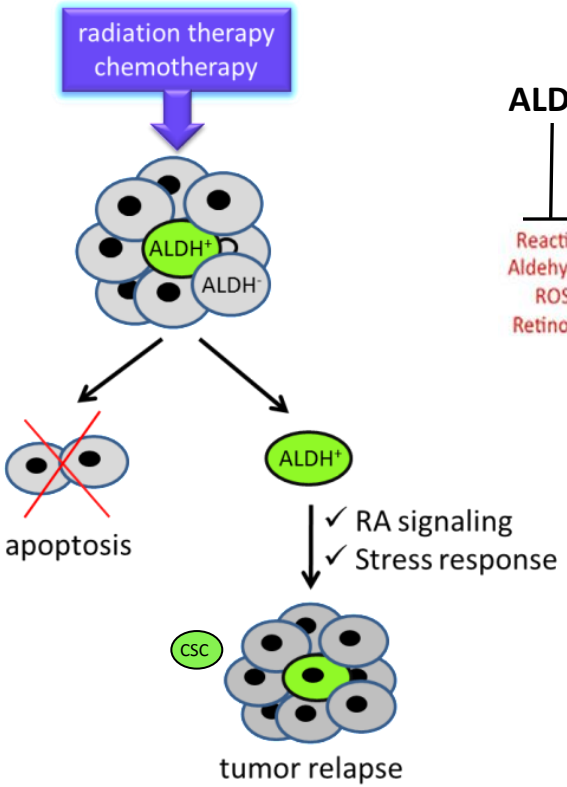
NCATS-Yale Collaboration on Aldehyde Dehydrogenase Inhibitors

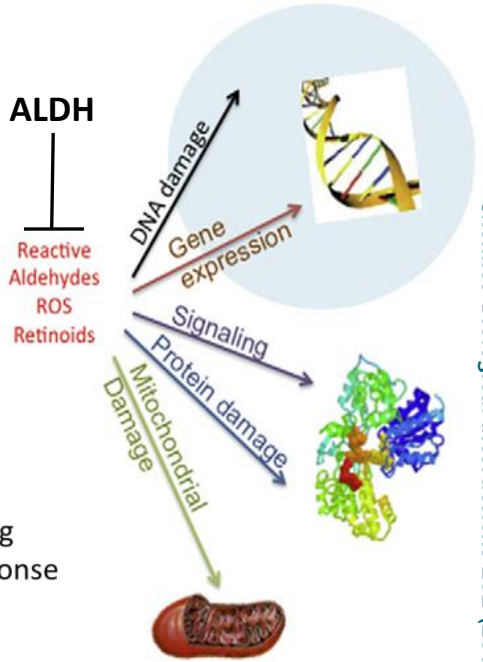
Collaborator: Vasilis Vasiliou, Yale University

Target: ALDH1A1

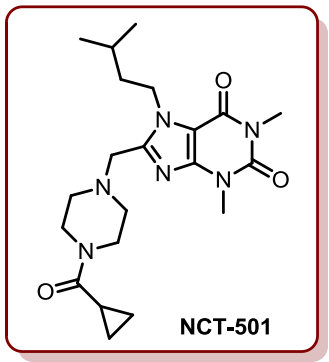
Therapeutic Scope: Cancer, Inflammation, Obesity, Development

Objective: Identification and characterization of small molecule inhibitors of Aldehyde Dehydrogenase isoform 1A1





Chemico-Biological Interactions 202 (2013) 2-10



Journal of Medicinal Chemistry

Article

pubs.acs.org/jmc

Discovery of NCT-501, a Potent and Selective Theophylline-Based Inhibitor of Aldehyde Dehydrogenase 1A1 (ALDH1A1)

Shyh-Ming Yang,[*,†] Adam Yasgar,[†] Bettina Miller,[‡] Madhu Lal-Nag,[†] Kyle Brimacombe,[†] Xin Hu,[†] Hongmao Sun,[†] Amy Wang,[†] Xin Xu,[†] Kimloan Nguyen,[†] Udo Oppermann,[§,∥] Marc Ferrer,[†] Vasilis Vasiliou,[‡,⊥] Anton Simeonov,[†] Ajit Jadhav,[†] and David J. Maloney[*,†]

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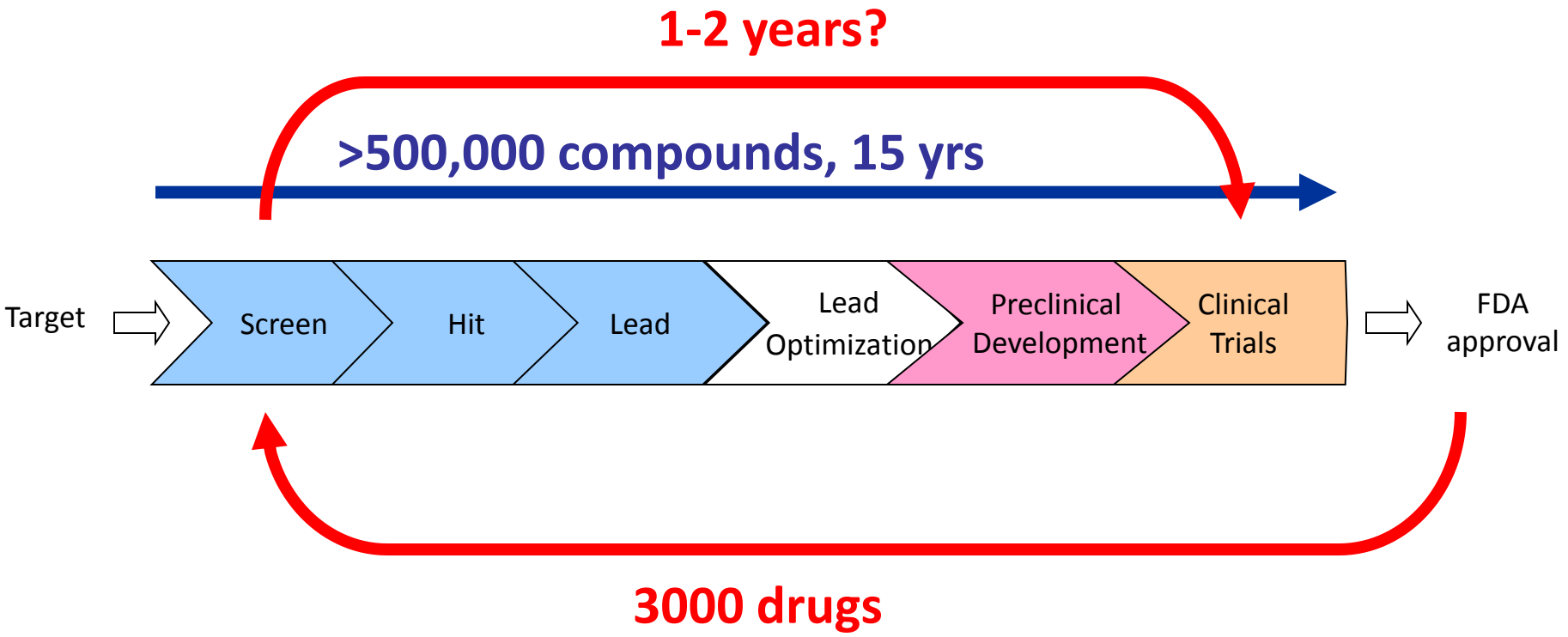
Drug Repurposing



NCATS Comprehensive Repurposing Program
"Systematizing Serendipity"

The NCGC Pharmaceutical Collection: A Comprehensive Resource of Clinically Approved Drugs Enabling Repurposing and Chemical Genomics

Ruili Huang,* Noel Southall,* Yuhong Wang, Adam Yasgar, Paul Shinn, Ajit Jadhav, Dac-Trung Nguyen, Christopher P. Austin†

Small-molecule compounds approved for use as drugs may be "repurposed" for new indications and studied to determine the mechanisms of their beneficial and adverse effects. A comprehensive collection of all small-molecule drugs approved for human use would be invaluable for systematic repurposing across human diseases, particularly for rare and neglected diseases, for which the cost and time required for development of a new chemical entity are often prohibitive. Previous efforts to build such a comprehensive collection have been limited by the complexities, redundancies, and semantic inconsistencies of drug naming within and among regulatory agencies worldwide; a lack of clear conceptualization of what constitutes a drug; and a lack of access to physical samples. We report here the creation of a definitive, complete, and nonredundant list of all approved molecular entities as a freely available electronic resource and a physical collection of small molecules amenable to high-throughput screening.

Patient-Driven Science

ACS chemical biology

Articles

pubs.acs.org/acschemicalbiology

Identification of Drug Modulators Targeting Gene-Dosage Disease CMT1A

Sung-Wook Jang,[†] Camila Lopez-Anido,[§] Ryan MacArthur,[†] John Svaren,[§] and James Inglese*[,†,‡]

[†]National Center of Advancing Translational Sciences and [‡]National Human Genome Research Institute, National Institutes of Health, Bethesda,

[§]Department of Cc

Cancer Biology & Therapy 14:7, 638–647; July 2013; © 2013 Landes Bioscience

Supporting In

ABSTRACT: T
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Each compound
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independent counter-screen for cytotoxicity, the design of our orthogon
prioritization of active compounds, among which three drugs (fenretini
of endogenous Pmp22 mRNA and protein. Overall, the findings of this
for gene-dosage diseases such as CMT1A.

Identification of repurposed small molecule drugs for chordoma therapy

Menghang Xia,[1,†,*] Ruili Huang,[1,†] Srilatha Sakamuru,[1] David Alcorta,[2] Ming-Hsuang Cho,[1] Dae-Hee Lee,[3] Deric M Park,[3] Michael J Kelley,[2] Josh Sommer,[4] and Christopher P Austin[1]

[1]NIH Chemical Genomics Center; Nati
[2]Department of Medicine; Duke University;

nature COMMUNICATIONS

Keywords: chordoma, NCGC

ARTICLE

Received 4 Mar 2013 | Accepted 23 May 2013 | Published 28 Jun 2013

DOI: 10.1038/ncomms3044

Induction and reversal of myotonic dystrophy type 1 pre-mRNA splicing defects by small molecules

Jessica L. Childs-Disney[1,*], Ewa Stepniak-Konieczna[2,*], Tuan Tran[1,3,*], Ilyas Yildirim[4], HaJeung Park[1], Catherine Z. Chen[5], Jason Hoskins[6], Noel Southall[5], Juan J. Marugan[5], Samarjit Patnaik[5], Wei Zheng[5], Chris P. Austin[5], George C. Schatz[4], Krzysztof Sobczak[2], Charles A. Thornton[6] & Matthew D. Disney[1]

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Partnering with Disease Foundations to Speed Drug Discovery

When scientists who specialize in drug development have a promising idea for a new disease treatment, they often start by designing biological tests called assays. By using high-throughput (robotically assisted) screening, researchers use the assays to evaluate hundreds of thousands of compounds with the potential to become new treatments. This complex process requires teamwork to involve the right types of expertise and perspectives in the research project team.

Designing high-throughput screening assays is a science in itself. The team must have in-depth familiarity not only with assay technology but also with the target disease and its unique challenges. When the disease is rare, limited information can present additional challenges.

As director of NCATS' Assay Development and Screening Technology Laboratory, Jim Inglese, Ph.D., leads a team of experts who take on these challenges every day. To increase the likelihood of success, Inglese encourages postdoctoral researchers who are knowledgeable about specific diseases to join project teams through fellowships sponsored by patient groups and foundations.

These fellows bring strong disease expertise to NCATS, where Inglese mentors them in broad translational capabilities including assay development and early drug discovery. The overall goal is to develop new technologies and methods to build better disease models that can help advance the search for potential treatments.

Hannah's Hope

Lori Sames, founder of Hannah's Hope Fund, and her daughter Hannah, who has giant axonal neuropathy, a progressive neurological condition. (Lori Sames Photo)

Charcot-Marie-Tooth Association

Michael J. Fox Foundation

Several of NCATS' Assay Development and Screening Technology Laboratory postdoctoral fellows (from left to right): Brittany Wright, Adam Fogel, Melissa Mendez and Michael Iannotti.

Hannah's Hope Fund

Alpha-1 Foundation

NIH National Center for Advancing Translational Sciences

http://ncats.nih.gov/pubs/features/adst-fellows

NCATS - Children Tumor Foundation (CTF) Collaboration

PIs: Annette Bakker (CTF), Jaishri Blakeley (JHU), Marc Ferrer (NCATS)

Disease focus: Neurofibromatosis 2 (NF2), characterized by multiple tumors on cranial and spinal nerves, and other lesions of the brain and spinal cord.

Goal: Discover new single drug and drug combination treatments for NF2.

Scope/Progress: CTF investigators (Synodos collaborative project funded by CTF) provided NCATS with NF2 Schwannomas and Meningiomas that have been screened with the NCATS MIPE 4.0 oncology collection (1,913 compounds). Selected compounds are being screened in dose response matrices to discover synergistic combinations.

The Learning Collaborative: Capitalizing on Strengths

KU THE UNIVERSITY OF KANSAS
The Institute for Advancing Medical Innovation

- Bench to bedside translation in drug repurposing
- National leadership in medicinal and pharmaceutical chemistry
- Pharma experience

NIH National Center for Advancing Translational Sciences

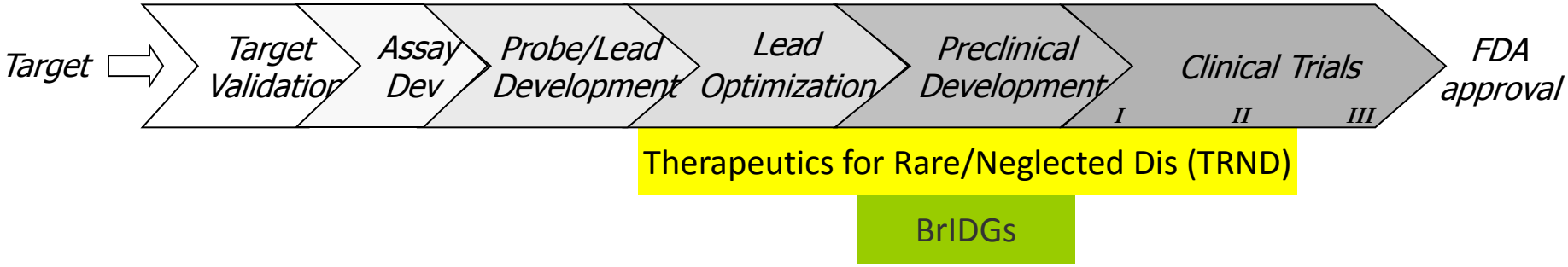
- Focus on rare and neglected diseases
- Industrial scale HTS, cheminformatics, medicinal chemistry, drug development capabilities
- Pharma experience

LEUKEMIA & LYMPHOMA SOCIETY
fighting blood cancers

- ~ 400 active research projects
- World-wide network of blood cancer experts
- Track record of commercial partnerships
- Pharma experience

NCATS Therapeutics Development Programs

Therapeutics for Rare and Neglected Diseases (TRND)
Bridging Interventional Development Gaps (BrIDGs)



Model: Collaboration between NCATS labs with preclinical drug development expertise and external organizations with disease area/target expertise

Projects:

Entry from Probe to IND-enabling

Exit by adoption by external organization for completion of clinical development

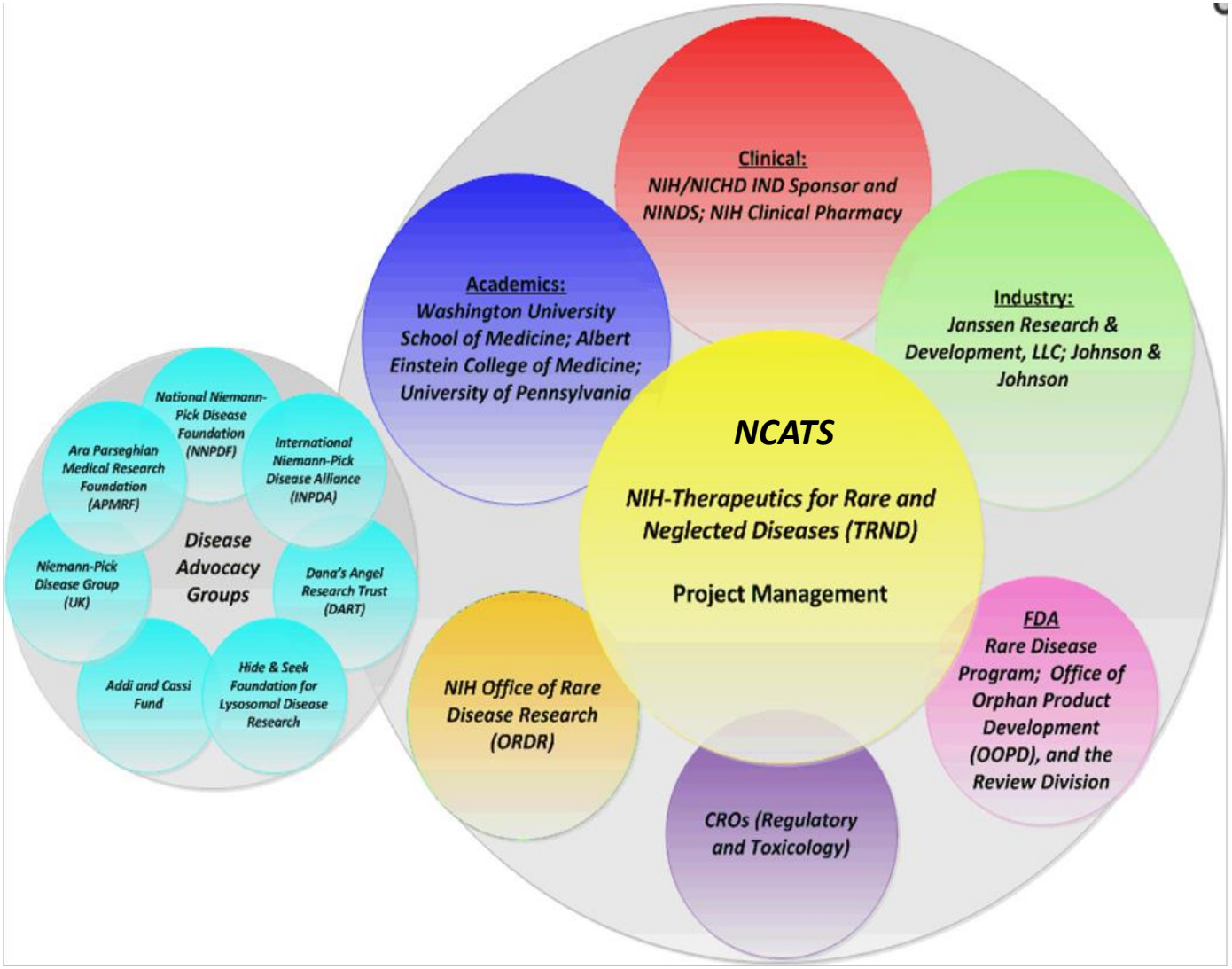
Serve to develop new generally applicable platform technologies and paradigms

Eligible Collaborators:

Academic, Non-Profit, Government Lab, Biotech, Pharma

Ex-U.S. applicants accepted

TRND Niemann-Pick C Disease Collaborative

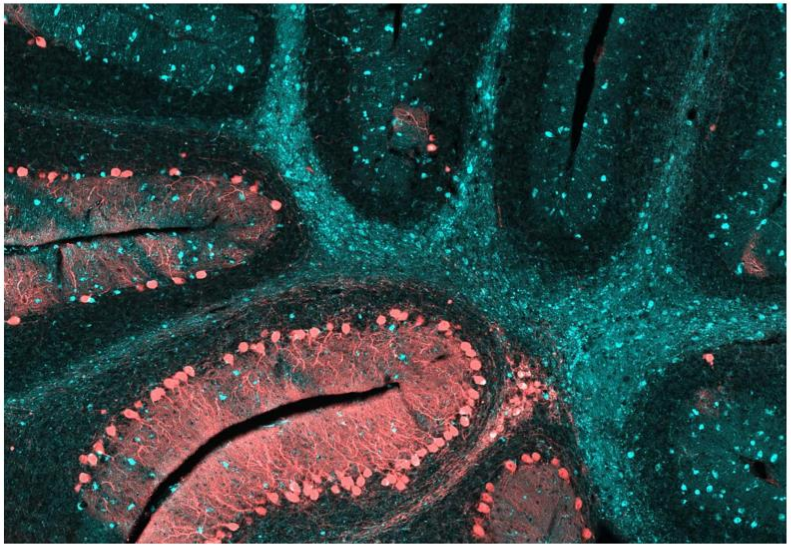


Ottinger et al., Curr Top Med Chem 2014;14(3):330

NEWS RELEASES

Wednesday, January 7, 2015

NIH teams with industry to develop treatments for Niemann-Pick Type C disease.



This image shows the cerebellum of a brain affected by NPC at the end stage of the disease. The blue staining shows the dense pockets of lipid accumulations throughout the brain. *NICHD*

Researchers from the National Institutes of Health have entered into an agreement with biotechnology company Vtesse, Inc., of Gaithersburg, Maryland, to develop treatments for Niemann-Pick disease type C (NPC) and other lysosomal storage disorders.

Lysosomal storage diseases, also known as lipid storage diseases, comprise about 50 rare inherited disorders that usually affect children. Fatty materials accumulate in the cells and tissues of the body. These diseases can result in damage to the brain, peripheral nervous system, liver, and other organs and tissues; they are often fatal.

Researchers at the National Center for Advancing Translational Sciences (NCATS) and the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), both parts of NIH, will conduct studies on NPC and other lysosomal storage disorders with funding provided by Vtesse.

"This is an excellent example of how launching a project to study the underlying biology of one disease can lead to advances that hold promise for an entire group of diseases — the NCATS goal of finding what is common among diseases and the translational science process," said NCATS Director Christopher P. Austin, M.D. "I am grateful to all of the NPC patients, their families and patient support groups who have been equal partners in our efforts to find therapeutic solutions to these devastating disorders."

> *"Our role is to test promising new drugs and therapies to ensure that they are safe and effective."*
>
> **—Forbes D. Porter, M.D., Ph.D.**
> NICHD Clinical Director

Institute/Center

Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)

National Center for Advancing Translational Sciences (NCATS)

Contact

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National Center for Advancing Translational Sciences

Tissue Chip Program

GOAL: Develop an *in vitro* platform that uses human tissues to evaluate the efficacy, safety and toxicity of promising therapies.

National Institutes of Health

Phase 1: Development

Phase 2: Cell incorporation & organ integration

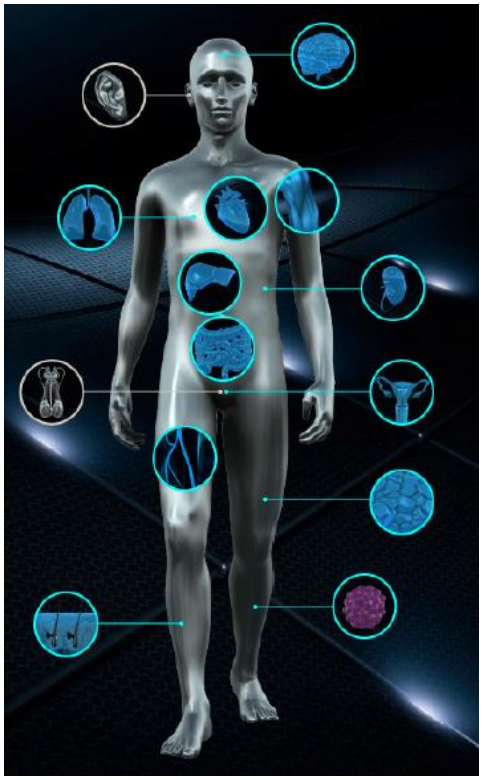
2012-13 | 2013-14 | 2014-15 | 2015-16 | 2016-17

DARPA

DARPA base periods: Organ integration

FDA

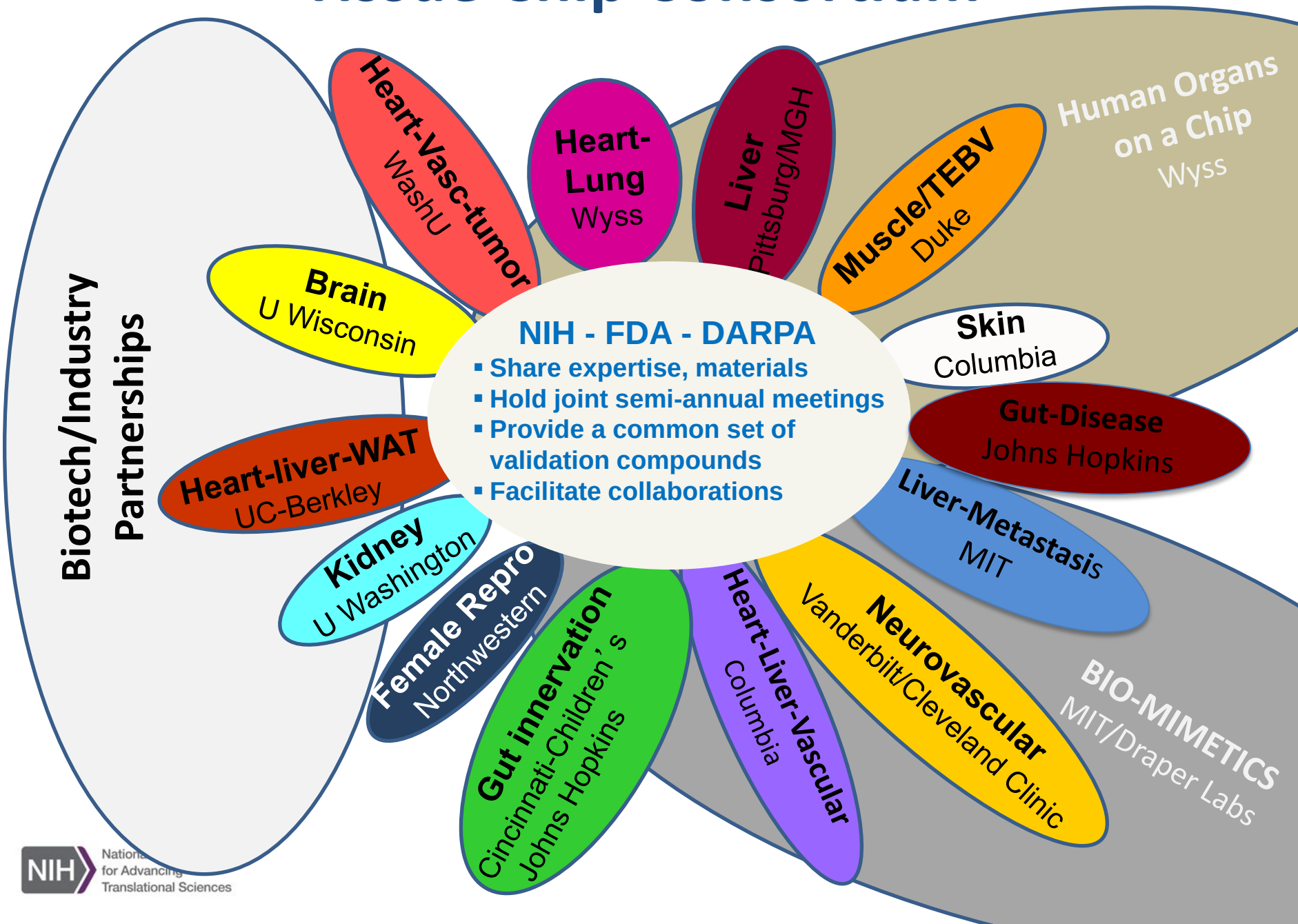
FDA providing insight and expertise throughout the program



Current Goals:

- Integration
- Compound testing
- Validation
- Partnerships
- Adoption by community

Tissue Chip Consortium



NIH National Institutes for Advancing Translational Sciences

Uniqueness of NCATS Portfolio

- Significant number of inventions relate to new therapeutic molecules and novel processes
- Inventions are much more advanced compared to other ICs
  - Indicators of high impact on facilitating translation of biomedical discoveries:
- High percentage of jointly owned inventions
  - Reflect the culture of NCATS aimed at using collaborations, alliances and partnerships to enhance positive outcomes and new cures

Snapshot of Inventive Activity at NCATS- Highly Collaborative Investigators

- Total Inventions: 130
- NCATS Solely Owned: 30
- Joint Inventions: 100
  - With other NIH Institutes: 37

  (NCI-CCR, NIDDK, NHGRI, NIAID, NHLBI, NIAMS, etc.)
  - With outside entities (majority academics): 63

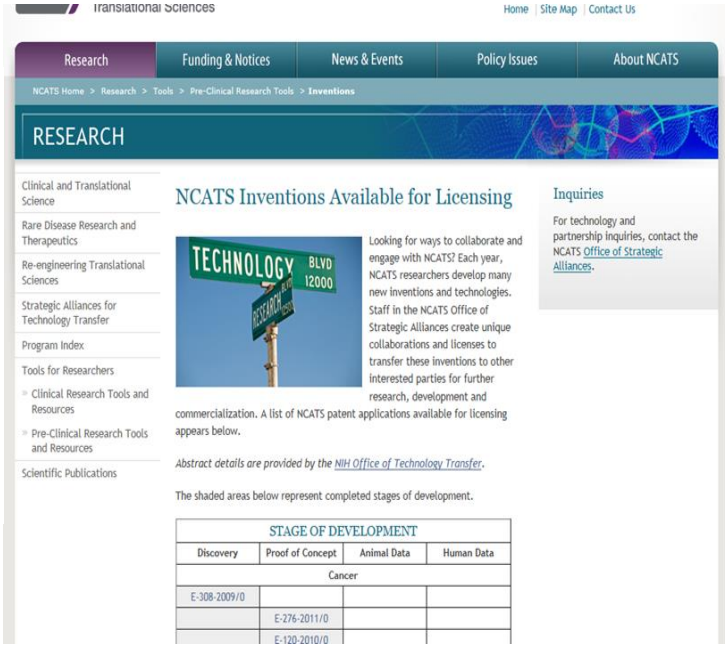
  (Boston University, Dana Faber, EVMS, FIU, Harvard, JHU, Loyola, MGH, Mt. Sinai, NYU, Northwestern, Rockefeller, U. Colorado, U. Delaware, U. Kansas, Karolinska, UMD, U. Mass, UNC, UPenn, UCSC, UCSD, U. Utah, U. Wisconsin, Vanderbilt,).

NCATS Licensing Webpage

http://www.ncats.nih.gov/licensing.html

STAGE OF DEVELOPMENT			
Discovery	Proof of Concept	Animal Data	Human Data
Cancer			
E-308-2009/0			
	E-276-2011/0		
	E-120-2010/0		
	E-326-2008/0		
	E-240-2011/0		
	E-148-2012/0		
	E-043-2013/0		
	E-300-2012/0		
		E-094-2011/0	
		E-298-2011/0	
Cardiovascular Diseases			
	E-072-2012/0		
	E-300-2012/0		
Diabetes			
E-256-2010/0			
	E-134-2010/0		
	E-300-2012/0		
		E-298-2011/0	
Infectious Diseases			
	E-162-2008/0		
	E-211-2010/2		
	E-300-2012/0		
Neurological Disorders			
E-230-2009/0			
E-041-2010/0			
	E-109-2010/0		
	E-148-2012/0		
	E-268-2009/0		
	E-070-2012/0		
	E-300-2012/0		
Metabolic and Storage Disorders			
	E-257-2010/0		
	E-326-2008/0		
	E-240-2011/0		
	E-277-2011/0		
	E-300-2012/0		
		E-298-2011/0	
Rare Diseases – Cancer			
	E-300-2012/0		

- Illustrates both Stage of Development and Indication
- Provides non-confidential information
- New format increased traffic by 250%
- Encourages discussion



NIH National Center for Advancing Translational Sciences

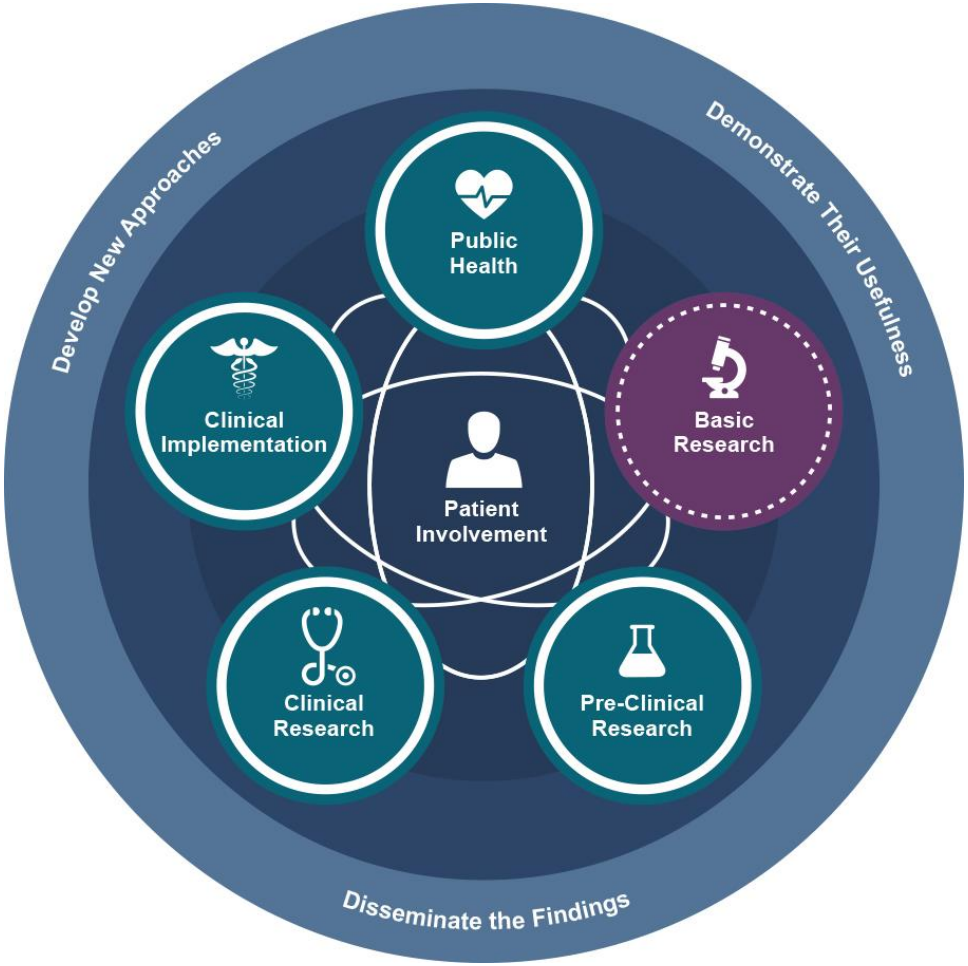
I-Corps™ Training Program at NCATS

- Developed at NSF: Innovation Corps (I-Corps™)
- Intensive *Entrepreneurial Immersion* course aimed at providing teams with skills and strategies to reduce commercialization risk
- Curriculum emphasizes ***Reaching out to Customers*** to test hypotheses about the need and market for the technology being developed.
  - Each team is expected to conduct over 100 interviews over 10 weeks.
- Format is focused on *Experiential Learning*

Arithmetic to Log-Phase Growth: NCATS CTSA I-Corps *Train-the Trainer* Program

- NCATS is training new I-Corps educators at CTSA institutions who in turn can provide entrepreneurship training for other translational scientists
- NCATS provided supplements to active CTSA grants to participate in the I-Corps Train-the Trainer Program
- 10 CTSAs were awarded supplements and are participating in the pilot program
- Plan is to expand program to other CTSAs utilizing feedback and learnings from pilot program

Translational Science Spectrum



Learn More About NCATS

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